Ascendis Pharma A/S Announces Target Enrollment Achieved in the Phase 3 PaTHway Trial of TransCon™ PTH (palopegteriparatide) in Adults with Hypoparathyroidism (HP) and Provides a Comprehensive Global Clinical Program Update

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– Ascendis expects to complete enrollment of subjects still in screening within one week –

– PaTHway top-line data now anticipated in Q1 2022 and submission of a New Drug Application (NDA) in mid-2022 –

– Announces design of PaTHway Japan Trial and receipt of Orphan Drug Designation (ODD) from the Japanese Ministry of Health, Labor and Welfare (MHLW) –

– Defined pathway for global regulatory filings –

COPENHAGEN, Denmark, July 06, 2021 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq: ASND), a biopharmaceutical company that utilizes its innovative TransCon technologies to create new product candidates that address unmet medical needs, today announced it has reached the target enrollment in the phase 3 PaTHway Trial, a trial evaluating the safety, tolerability, and efficacy of TransCon PTH (palopegteriparatide) in addition to providing a comprehensive global clinical program update.

“The durable benefit demonstrated in our phase 2 PaTH Forward Trial Week 58 data supports the use of TransCon PTH as a potential new treatment paradigm for adults with HP. We are committed to bringing TransCon PTH as quickly as possible to all patients with HP independent of etiology,” said Jan Mikkelsen, Ascendis Pharma’s President and Chief Executive Officer. “Given the strong demand and our commitment to patients and clinical investigators, although we stopped screening new subjects in early June, if any subject had started the screening process by June 7th, they will be enrolled in the phase 3 PaTHway Trial if all the criteria are met. We anticipate announcing top-line results from the PaTHway Trial in the first quarter of 2022, and if successful, followed by an expected NDA submission in mid-2022.”

“We are pleased to continue extending the global clinical reach for TransCon PTH for all HP patients as a key component of our Vision 3x3. With recent acceptances from regulatory authorities relating to the PaTHway Japan Trial and the PaTHway China Trial through VISEN Pharmaceuticals (VISEN), we believe we now have a defined pathway to obtain regulatory approval in countries representing over 400,000 patients,” continued Mr. Mikkelsen.

The PaTHway Trial is a phase 3, randomized, double-blind, placebo-controlled trial in North America and Europe evaluating the safety, tolerability, and efficacy of palopegteriparatide in adults with HP. The primary efficacy endpoint is the proportion of subjects with albumin-adjusted serum calcium within the normal range, and independent from active vitamin D and therapeutic doses of calcium (≤ 600 mg/day) at 26 weeks. If successful, Ascendis expects to submit an NDA to the U.S. Food and Drug Administration (FDA) in mid-2022 and subsequently submit a Marketing Authorisation Application to the European Medicines Agency.

Ascendis also announced the Japanese Pharmaceuticals and Medical Devices Agency (PMDA) acceptance of the clinical trial notification for the PaTHway Japan Trial, a single-arm, phase 3 trial of palopegteriparatide in a minimum of 12 Japanese subjects with HP. Subjects will start with an 18 µg dose of palopegteriparatide and be followed over a 26-week period during which they will be titrated to an optimal dose. In addition, MHLW granted ODD to palopegteriparatide for the treatment of HP. In Japan, ODD is granted to therapies intended for use in less than 50,000 patients in Japan and for which significant unmet medical need exists. The designation is granted by the MHLW based on the opinion of the Pharmaceutical Affairs and Food Sanitation Council.

In Greater China, VISEN recently announced that they obtained the IND approval from the Center for Drug Evaluation of the National Medical Products Administration for the phase 3 China clinical trial (PaTHway China Trial) of TransCon PTH on June 1, 2021 and is soon expected to initiate the study of TransCon PTH in patients with HP in China. The design of the PaTHway China Trial mirrors the design of the PaTHway Trial.

All subjects from phase 3 PaTHway trials in North America, Europe, Japan and Greater China will be eligible to enter into open-label extensions to collect long-term follow-up data.

Palopegteriparatide was granted ODD from the FDA in June 2018 and from the European Commission in October 2020.

About Ascendis Pharma A/S

Ascendis Pharma is applying its innovative platform technology to build a leading, fully integrated biopharma company focused on making a meaningful difference in patients’ lives. Guided by its core values of patients, science and passion, the company utilizes its TransCon technologies to create new and potentially best-in-class therapies.

Ascendis Pharma currently has a pipeline of three independent endocrinology rare disease product candidates and one oncology product candidate in clinical development. The company continues to expand into additional therapeutic areas to address unmet patient needs.

Ascendis is headquartered in Copenhagen, Denmark, with additional facilities in Heidelberg and Berlin, Germany, in Palo Alto and Redwood City, California, and in Princeton, New Jersey.

Please visit www.ascendispharma.com (for global information) or www.ascendispharma.us (for U.S. information).

About TransCon PTH[1]

TransCon PTH is an investigational once-daily long-acting prodrug of parathyroid hormone (PTH[1-34]) in development as a treatment for adult HP designed to restore PTH at physiologic levels for 24 hours each day to address both the short-term symptoms and long-term complications of the disease. TransCon PTH was granted ODD by the FDA in June 2018, in October 2020 was granted Orphan Designation by the European Commission for the treatment of HP, and in June 2021 was granted ODD by the MHLW.

About Hypoparathyroidism (HP) [2,3,4,5,6,7]

HP is a rare endocrine disorder characterized by insufficient levels of parathyroid hormone (PTH), resulting in low calcium and elevated phosphate levels in the blood. HP affects approximately 400,000 patients in the United States, Europe, Japan, South Korea and Greater China, the majority of whom develop the condition following damage or accidental removal of the parathyroid glands during thyroid surgery. Patients often experience decreased quality of life. In the short term, symptoms include weakness, severe muscle cramps (tetany), abnormal sensations such as tingling, burning and numbness (paresthesia), memory loss, impaired judgment and headache. Over the long term, this complex disorder can increase risk of major complications, such as extraskeletal calcium depositions occurring within the brain, lens of the eye, and kidneys, which can lead to impaired renal function.

HP remains among the few hormonal insufficiency states without a replacement therapy that restores the missing hormone at physiologic levels. Standard of care with active vitamin D analogs and calcium supplementation do not fully control the disease and may contribute to risk of renal disease. As a result, patients with HP have an estimated 4-fold to 8-fold greater risk of renal disease compared to healthy controls.

Forward-Looking Statements

This press release contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, included in this press release regarding Ascendis' future operations, plans and objectives of management are forward-looking statements. Examples of such statements include, but are not limited to, statements relating to (i) the timing of expected completion of enrollment in the phase 3 PaTHway Trial, (ii) the expected timing of reporting PaTHway top-line data and, if successful, the expected timing of a submission of an NDA and MAA for TransCon PTH, (iii) the eligibility of subjects from the phase 3 PaTHway trial in North America, Europe, Japan and Greater China to enter into open-label extensions to collect long-term follow-up data, (iv) Ascendis' ability to apply its platform technology to build a leading, fully integrated biopharma company, (v) Ascendis' product pipeline and expansion into additional therapeutic areas and (vi) Ascendis' expectations regarding its ability to utilize its TransCon technologies to create new and potentially best-in-class therapies. Ascendis may not actually achieve the plans, carry out the intentions or meet the expectations or projections disclosed in the forward-looking statements and you should not place undue reliance on these forward-looking statements. Actual results or events could differ materially from the plans, intentions, expectations and projections disclosed in the forward-looking statements. Various important factors could cause actual results or events to differ materially from the forward-looking statements that Ascendis makes, including the following: unforeseen safety or efficacy results in its oncology programs, TransCon hGH, TransCon PTH and TransCon CNP or other development programs; unforeseen expenses related to the development and potential commercialization of its oncology programs, TransCon hGH, TransCon PTH and TransCon CNP or other development programs, selling, general and administrative expenses, other research and development expenses and Ascendis' business generally; delays in the development of its oncology programs, TransCon hGH, TransCon PTH and TransCon CNP or other development programs related to manufacturing, regulatory requirements, speed of patient recruitment or other unforeseen delays; dependence on third party manufacturers to supply study drug for planned clinical studies; Ascendis' ability to obtain additional funding, if needed, to support its business activities and the effects on its business from the worldwide COVID-19 pandemic. For a further description of the risks and uncertainties that could cause actual results to differ from those expressed in these forward-looking statements, as well as risks relating to Ascendis' business in general, see Ascendis' Annual Report on Form 20-F filed with the U.S. Securities and Exchange Commission (SEC) on March 10, 2021 and Ascendis' other future reports filed with, or submitted to, the SEC. Forward-looking statements do not reflect the potential impact of any future in-licensing, collaborations, acquisitions, mergers, dispositions, joint ventures, or investments that Ascendis may enter into or make. Ascendis does not assume any obligation to update any forward-looking statements, except as required by law.

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